Stem Cell Agency Board Invests in 19 Discovery Research Programs Targeting Cancers, Heart Disease and Other Disorders

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South San Francisco, CA – While stem cell and gene therapy research has advanced dramatically in recent years, there are still many unknowns and many questions remaining about how best to use these approaches in developing therapies. That's why the governing Board of the California Institute for Regenerative Medicine (CIRM) today approved investing almost $25 million in 19 projects in early stage or Discovery research.

The awards are from CIRM's DISC2 Quest program, which supports the discovery of promising new stem cell-based and gene therapy technologies that could be translated to enable broad use and ultimately, improve patient care.

"Every therapy that helps save lives or change lives begins with a researcher asking a simple question, "What if?", says Dr. Maria T. Millan, the President and CEO of CIRM. "Our Quest awards reflect the need to keep supporting early stage research, to gain a deeper understanding of how stem cells work and how we can best tap into that potential to advance the field."

Dr. Judy Shizuru at Stanford University was awarded $1.34 million to develop a safer, less-toxic form of bone marrow or hematopoietic stem cell transplant (HCT). HCT is the only proven cure for many forms of blood disorders that affect people of all ages, sexes, and races worldwide. However, current methods involve the use of chemotherapy or radiation to destroy the patient's own unhealthy blood stem cells and make room for the new, healthy ones. This approach is toxic and complex and can only be performed by specialized teams in major medical centers, making access particularly difficult for poor and underserved communities.

Dr. Shizuru proposes developing an antibody that can direct the patient's own immune cells to kill diseased blood stem cells. This would make stem cell transplant safer and more effective for the treatment of many life-threatening blood disorders, and more accessible for people in rural or remote parts of the country.

Dr. Lili Yang at UCLA was awarded $1.4 million to develop an off-the-shelf cell therapy for ovarian cancer, which causes more deaths than any other cancer of the female reproductive system.

Dr. Yang is using immune system cells, called invariant natural killer T cells (iNKT) to attack cancer cells. However, these iNKT cells are only found in small numbers in the blood so current approaches involve taking those cells from the patient and, in the lab, modifying them to increase their numbers and strength before transplanting them back into the patient. This is both time consuming and expensive, and the patient's own iNKT cells may have been damaged by the cancer, reducing the likelihood of success.

In this new study Dr. Yang will use healthy donor cord blood cells and, through genetic engineering, turn them into the specific form of iNKT cell therapy targeting ovarian cancer. This DISC2 award will support the development of these cells and do the necessary testing and studies to advance it to the translational stage.

Timothy Hoey and Tenaya Therapeutics Inc. have been awarded $1.2 million to test a gene therapy approach to replace heart cells damaged by a heart attack.

Heart disease is the leading cause of death in the U.S. with the highest incidence among African Americans. It's caused by damage or death of functional heart muscle cells, usually due to heart attack. Because these heart muscle cells are unable to regenerate the damage is permanent. Dr. Hoey's team is developing a gene therapy that can be injected into patients and turn their cardiac fibroblasts, cells that can contribute to scar tissue, into functioning heart muscle cells, replacing those damaged by the heart attack.

The full list of DISC2 Quest awards is:

APPLICATION NUMBER	TITLE OF PROGRAM	PRINCIPAL INVESTIGATOR	AMOUNT
DISC2-13400	Targeted Immunotherapy-Based Blood Stem Cell Transplantation	Judy Shizuru, Stanford University	$1,341,910
DISC2-13505	Combating Ovarian Cancer Using Stem Cell-Engineered Off-The-Shelf CAR-iNKT Cells	Lili Yang, UCLA	$1,404,000
DISC2-13515	A treatment for Rett syndrome using glial-restricted neural progenitor cells	Alysson Muotri, UC San Diego	$1,402,240
DISC2-13454	Targeting pancreatic cancer stem cells with DDR1 antibodies.	Michael Karin, UC San Diego	$1,425,600
DISC2-13483	Enabling non-genetic activity-driven maturation of iPSC-derived neurons	Alex Savtchenko, Nanotools Bioscience	$675,000
DISC2-13405	Hematopoietic Stem Cell Gene Therapy for Alpha Thalassemia	Don Kohn, UCLA	$1,323,007
DISC2-13507	CAR T cells targeting abnormal N-glycans for the treatment of refractory/metastatic solid cancers	Michael Demetriou, UC Irvine	$1,414,800

DISC2-13463	Drug Development of Inhibitors of Inflammation Using Human iPSC-Derived Microglia (hiMG)	Stuart Lipton, Scripps Research Inst.	$1,658,123
DISC2-13390	Cardiac Reprogramming Gene Therapy for Post-Myocardial Infarction Heart Failure	Timothy Hoey, Tenaya Therapeutics	$1,215,000
DISC2-13417	AAV-dCas9 Epigenetic Editing for CDKL5 Deficiency Disorder	Kyle Fink, UC Davis	$1,429,378
DISC2-13415	Defining the Optimal Gene Therapy Approach of Human Hematopoietic Stem Cells for the Treatment of Dedicator of Cytokinesis 8 (DOCK8) Deficiency	Caroline Kuo, UCLA	$1,386,232
DISC2-13498	Bioengineering human stem cell-derived beta cell organoids to monitor cell health in real time and improve therapeutic outcomes in patients	Katy Digovich, Minutia, Inc.	$1,198,550
DISC2-13469	Novel antisense therapy to treat genetic forms of neurodevelopmental disease.	Joseph Gleeson, UC San Diego	$1,180,654
DISC2-13428	Therapeutics to overcome the differentiation roadblock in Myelodysplastic Syndrome (MDS)	Michael Bollong, Scripps Research Inst.	$1,244,160

DISC2-13456	Novel methods to eliminate cancer stem cells	Dinesh Rao, UCLA	$1,384,347
DISC2-13441	A new precision medicine based iPSC-derived model to study personalized intestinal fibrosis treatments in pediatric patients with Crohn's disease	Robert Barrett Cedars-Sinai	$776,340
DISC2-13512	Modified RNA-Based Gene Therapy for Cardiac Regeneration Through Cardiomyocyte Proliferation	Deepak Srivastava, Gladstone Institutes	$1,565,784
DISC2-13510	An hematopoietic stem-cell-based approach to treat HIV employing CAR-T cells and anti-HIV broadly neutralizing antibodies	Brian Lawson, The Scintillon Institute	$1,143,600
DISC2-13475	Developing gene therapy for dominant optic atrophy using human pluripotent stem cell-derived retinal organoid disease model	Xian-Jie Yang, UCLA	$1,345,691

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is one of the world's largest institutions dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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